ACCELERATE
INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

Programme

8-9 February 2024
Brussels & Online

Tangla Hotel Brussels

ANDREW McDONOUGH B+ FOUNDATION

Day 1 - February 8
Time CET

09:00 - 10:00 — **Registration**

10:00 - 10:45 — **Welcome and Keynote address**

10:00 - 10:10
- **Welcome and introduction** - Gilles Vassal, *ACCELERATE Chair*

10:10 - 10:35
- **Keynote talk on research in GD2 CAR T-cells in Neuroblastoma** - Franco Locatelli, *IRCCS Bambino Gesù Children's Hospital*

10:35 - 10:45
- **Q&A**

10:45 - 12:30 — **Session I – New Business Models to Accelerate Innovation in Pediatric Oncology Drug Development**

Chair: Pamela Kearns, *University of Birmingham*

10:45 - 11:00
- **Setting the scene** - Gilles Vassal, *ACCELERATE Chair*

11:00 - 11:15
- **New business model for investors, private companies and public organizations to invest in drug development for paediatric cancers -** Sam Daems, *Waterland Private Equity Investments, Antwerp and Institute, Université libre de Bruxelles, Belgium*

11:15 - 12:30
- **Multi-Stakeholder panel discussion** - Patricia Blanc, *Imagine for Margo*, David Jenkinson, *Life Arc*, Alessandro Aiuti, *Fondazione Telethon*, Thomas Rooney, *Sanofi,* Sam Daems*, ULB*

12:30 - 13:30 — **Lunch**

13:30 - 15:30 — **Session II – Regulatory update and ACCELERATE achievements**

Chair: Leona Knox, *Solving Kids Cancer UK*

13:30 - 14:00
- **European Medicines Agency Keynote and Regulatory update in the US** - Steffen Thirstrup, *European Medicines Agency* & Martha Donoghue, *U.S. Food and Drug Administration*

14:00 - 14:20
- **ACCELERATE Logbook** - Kersin Sollerbrant, *The Swedish Childhood Cancer Fund* & Andy Pearson, *ACCELERATE*

14:20 - 14:40
- **FAIR Working Group**
  - Update of the group activities - Max Williamson, *ACCELERATE FAIR WG Co-chair*
  - Age inclusion and relevance of approved cancer medicines in Europe and USA for AYA - Ines Alves, *ACCELERATE*

14:40 - 14:55
- **Patient-Reported Outcomes** - David Riedl, *Innsbruck University*

14:55 - 15:15
- **ALADDIN** - Chair: Cornelis van Tilburg, *KITZ Heidelberg*
  - Update - Teresa de Rojas, *ACCELERATE and ALADDIN Coordinator*
  - Participants' experience and panel discussion - Teresa de Rojas, *ALADDIN* Coordinator Advanced Course students - Pablo Velasco, *Vall d'Hebron Hospital*, Alan Pearson, *Childhood Cancer Ireland,* Erica Brivio, *Princess Máxima Center*

15:15 - 15:30
- **Advocate workshop report** - Nick Bird, *Solving Kids Cancer UK*

  The scientific and moral dilemma of randomising hope in poor prognosis children's cancers

15:30 - 16:00 — **Coffee break & group photo**

16:00 - 16:30 — **Paediatric Strategy Forums: present and future** - Andy Pearson, *ACCELERATE*

16:30 - 18:15 — **Session III – Parallel Breakout Sessions**
- **Topic 1.** Improved access to investigational drugs for academia to conduct clinical trials
- **Topic 2.** Optimizing decentralized elements to improve access to clinical trials exploring new medicinal products for paediatric patients with cancer
- **Topic 3.** How can the concept of "prioritization" of products be concretely developed and applied in practice?

18:15 — **End of Day 1**

20:00 — **Networking dinner**

Day 2 - February 9
Time CET

08:00 - 08:30 — **Registration**

08:30 - 10:30 — **Session IV – International academic trials for paediatric drug development**
Chair: Douglas Hawkins, *COG Chair, Seattle Children's Hospital*

- **08:30 - 08:45** — **Experience in Europe** - Lynley Marshall, *The Royal Marsden*
- **08:45 - 09:00** — **Experience in the United States** - Elizabeth Fox, *St. Jude Children's Research Hospital*
- **09:00 - 09:15** — **Fit for filing experience** - Michel Zwaan, *Princess Maxima Center*
- **09:15 - 09:30** — **ITCC-COG CTEP international trial: enhancing academic collaboration -** Pamela Kearns, *University of Birmingham*
- **09:30 - 09:45** — **Accelerating Clinical Trials in Europe – ACT EU -** Kim Pietsch, *European Medicines Agency*
- **09:45 - 10:30** — **Multi-Stakeholder panel discussion -** Lynley Marshall, *The Royal Marsden*, Elizabeth Fox, *St. Jude Children's Research Hospital*, Michel Zwaan, *Princess Maxima Center*, Pamela Kearns, *University of Birmingham*, Anjali Sharma, *Gilead Sciences, Inc.*, Kim Pietsch, *EMA*

10:30 - 11:00 — **Coffee break**

11:00 - 12:45 — **Session V – Improved academia access to investigational drugs and optimizing decentralized elements to improve access to clinical trials**
Chair: Mark Kieran, *Day One Biopharmaceuticals*

- **11:00 - 11:15** — **Rescuing drugs that are discontinued in adult oncology** - Davy Chiodin, *Day One Biopharmaceuticals*
- **11:15 - 12:00** — **Report from Breakout Session 1** "Improved access to investigational drugs for academia to conduct clinical trials" and discussion
- **12:00 - 12:45** — **Report from Breakout Session 2** "Optimizing decentralized elements to improve access to clinical trials exploring new medicinal products for paediatric patients with cancer" and discussion

12:45 - 13:45 — **Lunch**

13:45 - 15:55 — **Session VI – How can the concept of "prioritization" of products be concretely developed and applied in practice?**
Chair: Julia Glade Bender, *MSK Kids*

- **13:45 - 14:00** — **Has ACCELERATE achieved its prioritization goals? -** Brenda Weigel, *University of Minnesota*
- **14:00 - 14:25** — **From vision to reality – reflections on the concept of prioritisation -** Dominik Karres, *European Medicines Agency*
- **14:25 - 14:55** — **Report from Breakout Session 3** "How can the concept of "prioritization" of products be concretely developed and applied in practice?" and discussion
- **14:55 - 15:55** — **Multi-Stakeholder panel discussion -** Andy Pearson, *ACCELERATE*, Brenda Weigel, *University of Minnesota*, Anjali Sharma, *Gilead*, Scott Diede, *MSD*, Donna Ludwinski, *Solving Kids Cancer*, Dominik Karres, *European Medicines Agency,* Angelika Joos, *EFPIA*

15:55 - 16:30 — **Wrap-up and end of conference**

Parallel Breakout Sessions

1. Improved access to investigational drugs for academia to conduct clinical trials. In order to conduct academic trials, researchers need access to investigational drugs.

Chairs Group 1: Steve DuBois, *Dana Farber Cancer Institute* & Nick Bird, *Solving Kids Cancer UK*
Chairs Group 2: Chinyere Okpara, *Eisai* & Lia Gore, *Children's Hospital Colorado*

There are increasing difficulties which slow, or even impair, access.

- What are the sources of the problem?
- What can be done to improve/accelerate access to investigational drugs for academic trials?

2. Optimizing decentralized elements to improve access to clinical trials exploring new medicinal products for paediatric patients with cancer.

Chairs Group 1: Kathy Brodeur-Robb, *C17 Council* & Nicole Scobie, *Zoe4Life*
Chairs Group 2: Paco Bautista, *Princess Máxima Center* - Angelika Joos, *MSD*

Clinical trials have recruitment problems (lack of “access” to the right children for the trial) and also children who need more options and could potentially benefit from new drugs in clinical trials cannot access trials. Now regulators are producing guidelines for decentralized elements to improve access to clinical trials.

- What elements can be decentralized in clinical trials exploring activity and safety of new medicinal products in children and adolescents with cancer?
- How to implement?
- How to measure impact?

3. How can the concept of "prioritization" of products be concretely developed and applied in practice?

Chairs Group 1: Julia Glade-Bender, *MSK Kids* & Anjali Sharma, *Gilead*
Chairs Group 2: Brenda Weigel, *University of Minnesota* & Scott Diede, *MSD*

In a landscape of mechanism of action driven drug development, both class and product prioritisation are necessary. Paediatric Strategy Forums have been successful in facilitating class prioritisation but it appears that neither Paediatric Strategy Forums nor subsequent Prioritization meetings have been so effective with regard to implementing product prioritisation. Multistakeholder prioritization is proposed by the European Commission in its proposal for revising the General Pharmaceutical legislation.

- What are the challenges for real Product prioritisation?
- What are the conditions for real and successful Product prioritisation?
- Deliver more than one concept proposal for implementing product prioritization.